

WO 2004/083430 A2

SSO 152

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization International Bureau



(43) International Publication Date  
30 September 2004 (30.09.2004)

PCT

(10) International Publication Number  
WO 2004/083430 A2

(51) International Patent Classification<sup>7</sup>: C12N 15/11, C07H 21/04, A61K 31/713, A61P 35/00

(74) Agent: PLOUGMANN & VINGTOFT A/S; Sundkrosgade 9, P.O. Box 831, DK-2100 Copenhagen Ø (DK).

(21) International Application Number:

PCT/DK2004/000192

(22) International Filing Date: 22 March 2004 (22.03.2004)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

PA 2003 00442	21 March 2003 (21.03.2003)	DK
60/456,888	21 March 2003 (21.03.2003)	US
PA 2003 01625	31 October 2003 (31.10.2003)	DK
PA 2004 00145	30 January 2004 (30.01.2004)	DK

(71) Applicant (for all designated States except US): SAN-TARIS PHARMA A/S [DK/DK]; Bøge Allé 3, DK-2970 Hørsholm (DK).

(72) Inventors; and

(75) Inventors/Applicants (for US only): ELMÉN, Joacim [SE/SE]; Olof af Acrelsväg 3, 6tr, SE-171 64 Solna (SE). WAHLESTEDT, Claes [SE/SE]; Strandvägen 29, SE-114 56 Stockholm (SE). LIANG, Zicai [SE/SE]; Sevensvägen 4, SE-174 46 Sundbyberg (SE). SØRENSEN, Anders, Malling [DK/DK]; Gråstensgade 3, 1. tv., DK-1677 København V (DK). ØRUM, Henrik [DK/DK]; Vil-drosevej 3, DK-3500 Værløse (DK). KOCH, Troels [DK/DK]; Funkiavej 47, DK-2300 Copenhagen S (DK).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARipo (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

— without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: SHORT INTERFERING RNA (siRNA) ANALOGUES

(57) Abstract: The present invention is directed to novel double-stranded short interfering (siRNA) analogues comprising locked nucleic acid (LNA) monomers. Such compounds induces sequence-specific post-transcriptional gene silencing in many organisms by a process known as RNA interference (RNAi). The compounds disclosed herein has improved properties compared to non-modified siRNAs and may, accordingly, be useful as therapeutic agents, e.g., in the treatment of various cancer forms. More particularly, the present invention is directed to siRNA analogues comprising a sense strand and an antisense strand, wherein each strand comprises 12-35 nucleotides and wherein the siRNA analogues comprise at least one locked nucleic acid (LNA) monomer.